Medicines & Healthcare products
Regulatory Agency

Innovative Licensing & Access Pathway (ILAP)
A streamlined approach to regulation and patient access

CDDF Annual Conference 2022 – Dr Dan O'Connor - MHRA



What is the ILAP?

- The ambition is to deliver safe, early and financially sustainable patient access to innovative medicines
- Key aspect is the partnership between the MHRA and three UK HTA bodies; NICE, SMC and AWTTC; and patient input
- Alignment of evaluation and access activities throughout the development pathway – integration of regulation and HTA
- The NHS in England and Scotland are closely engaged, along with the Accelerated Access Collaborative and other UK health system partners

Innovative Licensing and Access Pathway overview

- **Innovation Passport:** A new medicine designation links to the development of a roadmap to patient access

- **Target Development Profile** (TDP): Creates a unique UK roadmap, utilising tools from a toolkit and providing a platform for sustained multi-stakeholder collaboration

- **A toolkit**: tools are intended to drive efficiencies in the development programme, supporting data generation and evidence requirements

- **An integrated pathway**: Pulls together expertise from across the MHRA, NICE, SMC and AWTTC partners in the wider healthcare system including the NHS in England and Scotland, patient experts

Innovation Passport (IP)

- Enables access to the pathway and future activities in the TDP:
    - Built-in flexibility, with multiple entry points along the pathway, apply with non-clinical data or clinical trial evidence, Commercial or non-commercial applicant

Principles

- Broad and inclusive definition of innovation in order to capture a wide range of products, including drug repurposing
- Non-clinical entry point provides ambition for long-term interactions
- Thinking about the patient from the start
- Encourages structured engagement between the MHRA, HTA and drug developer
- Joint decision making between MHRA, NICE, SMC and AWTTC and patient input (reference group)
- 3 criteria, all should be met for a positive opinion – important - potential for benefit to patients

Innovation Passport – criteria to be met

Criterion 1: Condition

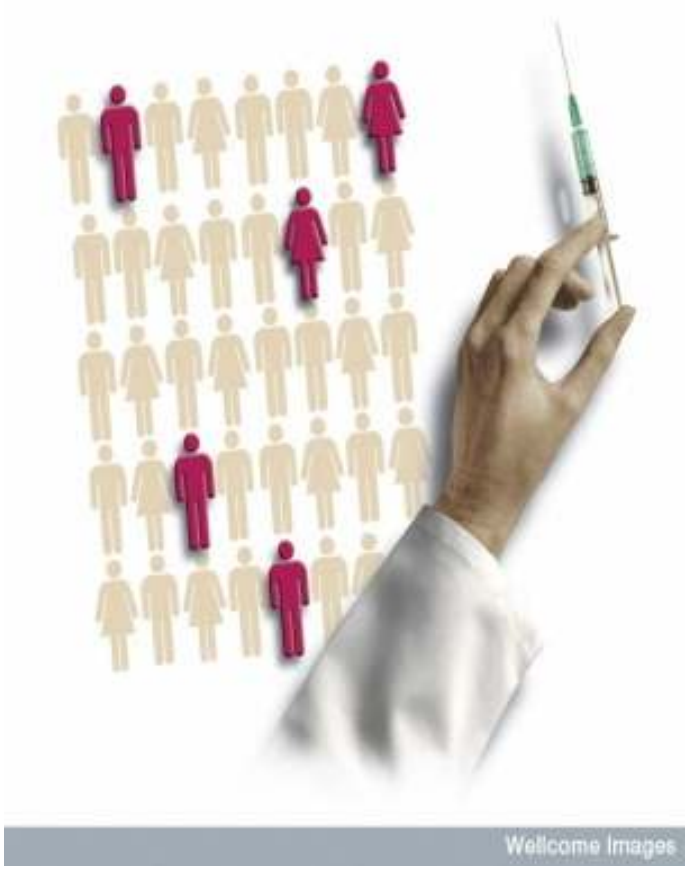
- The condition is life-threatening or seriously debilitating or
- There is a significant patient or public health need

Criterion 2: Type of medicine

- Innovative medicine (ATMP, first in class or novel drug device combination)
- Clinically significant new indication
- Medicine for rare disease and/or other special populations e.g. children, elderly, pregnant women
- Development in line with objectives for public health priorities

Criterion 3: Medicine has the potential to offer benefits to patients

- improved efficacy or safety, contribution to patient care or quality of life, as compared to alternative therapeutic options



ILAP IP activity 2021

- 76 applications (Oncology 25, neurology 13, respiratory 7), 44 granted, 23 pending, 8 refused, 1 withdrawn

- Variety of sized companies – large and small, spinout from UK university
- Includes products for FDA Orbis in oncology (11 IP applications expressed specific interest)
- Rare and common diseases

- First Innovation Passport
- Issued for treatment developed in a rare condition

  - von Hippel Lindau disease

Press release

First Innovation Passport awarded to help support development and access to cutting-edge medicines

The Innovative Licensing and Access Pathway (ILAP) aims to reduce the time to market for innovative medicines

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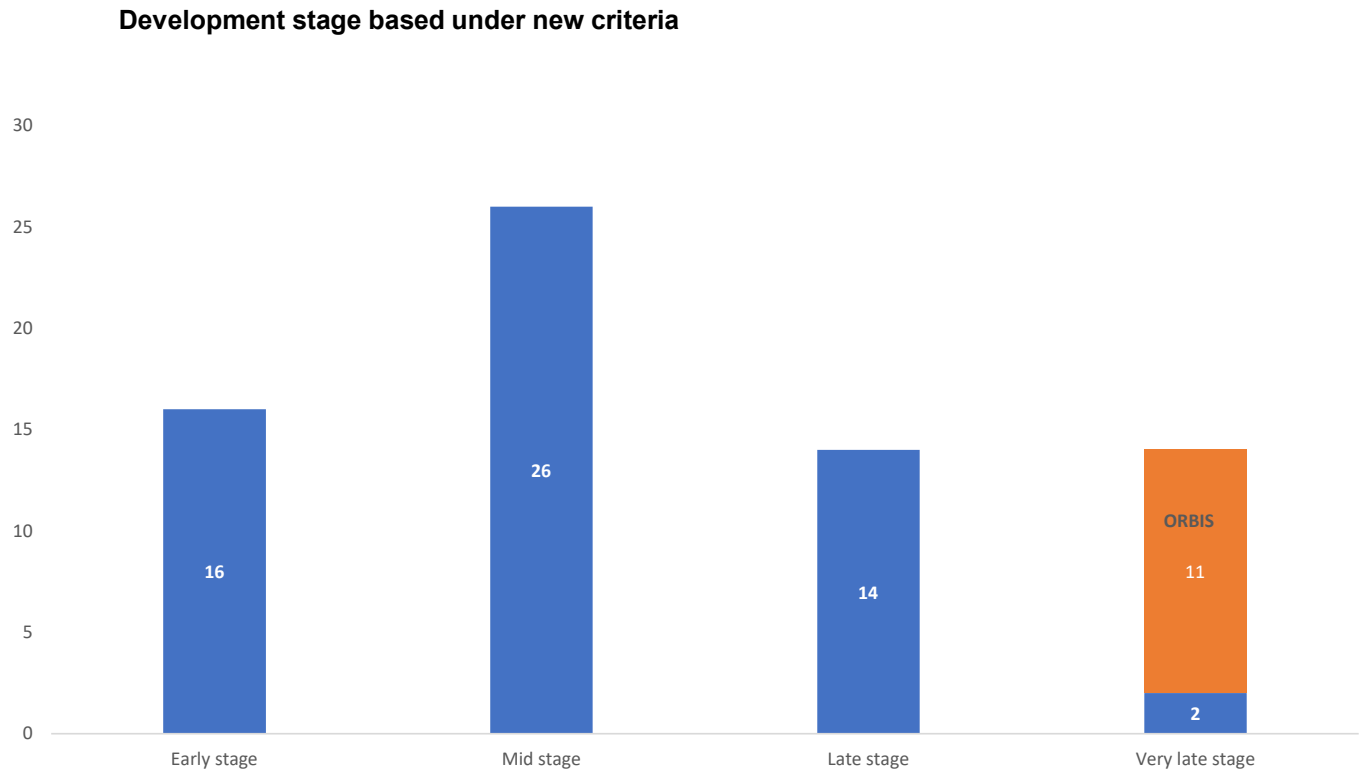
MHRA

A promising treatment for a cancer-causing rare disease will be the first to pass a significant milestone under a new UK approval process designed to bring medicines more rapidly to patients.

Belzutifan, a treatment developed by MSD (UK) for adults with von Hippel Lindau disease (a rare genetic disorder that causes cancer) has been awarded the first 'Innovation Passport' by the Medicines and Healthcare products Regulatory Agency, National Institute for Health and Care Excellence and the Scottish Medicines Consortium (SMC).

IP applications by development stage



Early stage:

First in human study not yet initiated, no clinical data available

Mid Stage

Phase 1 and or non-confirmatory phase 2 clinical trials ongoing or conducted, pre-phase 3 (not recruiting), early clinical data available on efficacy and safety

Late Stage

Confirmatory phase 2 or phase 3 trial on going or completed, significant clinical data available

Very Late Stage

Imminent Marketing Authorisation (MA) submission

Target Development Profile (TDP) Roadmap

- **TDP will define key regulatory and development features, identify potential pitfalls and create a road map for delivering early patient access, including tools from a toolkit**

ILAP TOOLKIT

- **The TDP step allows high-level consideration of a broad range of issues impacting product development, licensing and access allowing end to end planning**

Tools being developed in the Toolkit

- Adaptive inspections
- Certifications
- Continuous benefit-risk assessmen that integrate real word evidence
- CPRD assisted recruitment in clinical trials
- nhanced patient engagemen
- New licensing procedures:
  - Rolling review
  - Accelerated timetables for marketing authorisation flexibilities
  - International options
- Novel CT methodology & design support
- Rapid Clinical Trial Dossier pre-assessment service
- Centre Accreditation
- CPRD control groups
- Common medicine & device trial design
- UK HTA Access Forum
- Coordinated approvals process for co-developed medicines & IVDs

What does ILAP mean for patients, their families and carers?

- ✓ Dedicated ILAP Patient and Public Reference Group
- ✓ Safe and timely access to innovative medicines addressing patient needs
- ✓ Patients ‘front and centre’ of the pathway including input to the decisions to grant the Innovation Passport
- ✓ Opportunity to shape clinical trials to be focussed on what matters to patients
- ✓ Potential to benefit patients beyond the UK

What does ILAP mean for the life sciences industry?

- ✓ Opportunity to engage earlier in the design of innovative and efficient trial designs, focusing on patient need and reducing clinical trial timelines and costs
- ✓ Early platform dialogue on evidence requirements across regulatory and HTA needs at the TDP and beyond (tools of the toolkit)
- ✓ Expedited regulatory routes leading to earlier market access (including international options)
- ✓ Early access planning including evidence development, managed access and commercial considerations
- ✓ Opportunity to plan at a UK level, with access to UK-wide HTA agencies and opportunities to engage with NHS payers across the UK

What does ILAP mean for the MHRA, UK HTA agencies and UK payers?

- ✓ Opportunity to collaborate, align and do our jobs better in facilitating safe, early and financially sustainable patient access to important medicines
- ✓ Alignment of evidence requirements, including planning for real world evidence collection where needed, joint decision making at the IP designation
- ✓ Common and early understanding of regulatory and access plans increases the opportunities to align regulatory, HTA and commissioning timelines
- ✓ Timely engagement allows opportunities to develop creative commercial and managed access approaches where needed

AWTTC

Medicines & Healthcare products Regulatory Agency

NICE National Institute for Health and Care Excellence

Healthcare Improvement Scotland | SMC

Future development of the ILAP

- New digital offer – marked improvements for those submitting applications for IP and TDP
- Additional guidance and further information via updated webpage
- Develop case studies to highlight benefits of engaging with ILAP
- New HTA Access tool, continued development and process improvement of the other tools of the toolkit
- Further collaboration with UK life sciences ecosystem to drive the end to end process e.g. delivery of clinical trials
- Encourage developers of genomic medicines to enter the pathway
- Building on the lessons learned for consideration of a medical devices pathway

How will the ILAP digital solution benefit Product Developers?

- I have an easy and simple end to end Digital experience of the application process
- I have less need for manual handling, and specific fields are auto-populated for me based on previous submissions
- I receive regular notifications at key stages of my application process
- I have transparency of application status through a single front door rather than offline email communication
- I'm able to withdraw applications through the Digital Solution with a click of a button
- I'm able to use previous submissions as templates and make simple changes to submit new applications
- I can upload supporting documents with scientific data directly within the system for my submission

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